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Editorial

Adeno-associated virus vectorology and gene therapy applications

The adeno-associated virus (AAV) vector system is a good approach to study gene function *in vivo*. Over the past decade, the vector has proven versatile in studying a variety of target genes, target organ systems, and disease states in a reproducible manner. There is no known pathology associated with AAV, and unless one emerges, the increased use of these vectors is expected because the transgene expression is long-term and apparently safe. This issue includes up-to-date information on the production of vectors based on AAV-2 and other serotypes and also non-AAV parvoviral vectors. With different infection patterns in different target tissues, it appears that these vectors can be used as "horses for courses." Tricks and tips for injecting AAV vectors into the brain and spinal cord, including the neonatal brain, are discussed extensively, as are results

in the brain with a variety of promoters including regulatable systems. Gene transfer strategies for the brain, spinal cord, eye, and heart are detailed with an emphasis on gene therapy for neurodegenerative diseases and stroke. Methods are also included for unbiased quantification of cell populations, either those expressing a reporter gene or those for an endogenous marker that is affected by gene transfer. In the era following the Human Genome Project, the power of the AAV vector system for functional genomics and target validation is "infectious," and it is hoped that the articles in this issue will aid scientists who wish to adopt these methods for their studies.

Ronald L. Klein
Guest Editor

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